

GLIAL CELL LINE-DERIVED NEUROTROPHIC FACTOR FOR THE TREATMENT OF NEURODEGENERATIVE DISEASES AND DIABETES

SUMMARY

The National Institute on Drug Abuse (NIDA) is seeking interested parties to license or co-develop GDNFOS peptides and non-coding RNAs as therapeutic agents for neurodegenerative diseases.

REFERENCE NUMBER

E-044-2012

PRODUCT TYPE

- Therapeutics

KEYWORDS

- Parkinson's disease
- ALS
- Multiple Sclerosis
- Glial Cell Line Derived Neurotrophic Factor (GDNF)
- GDNFOS3.

COLLABORATION OPPORTUNITY

This invention is available for licensing and co-development.

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DESCRIPTION OF TECHNOLOGY

The [National Institute on Drug Abuse](https://www.nida.nih.gov/) (NIDA) is seeking interested parties to license or co-develop GDNFOS peptides and non-coding RNAs as therapeutic agents for neurodegenerative diseases.

Glial cell line-derived neurotrophic factor (GDNF) is a small human protein encoded by the GDNF gene. GDNF has been effective therapy in laboratory animal models of Parkinson's disease and protects several types of neurons in the brain and peripheral nervous system. Researchers at the NIDA have discovered primate-specific GDNFOS, encoded by the opposite strand of glial cell derived neurotrophic factor (GDNF) gene. The GDNFOS gene encodes for novel peptides. These secreted growth proteins have potential neurotrophic activity and were found to be reduced in the middle temporal gyrus of Alzheimer's disease patients, and they might play a synergistic role in neuroprotective effects of GDNF in

the human brain. The NIDA inventors have also developed an antibody against GDNFOS3 and generated compounds that have potential pharmaceutical use. The compounds consist of GDNFOS nucleic acid transcripts, GDNFOS protein or a functional fragment for treatment of human neurodegenerative diseases.

POTENTIAL COMMERCIAL APPLICATIONS

- Synergistic role in neuroprotective effects of GDNF
- Alzheimer's disease, Parkinson's disease,
- Amyotrophic lateral sclerosis, multiple sclerosis
- Diseases of peripheral organs such as diabetes mellitus type 1

COMPETITIVE ADVANTAGES

- Secreted novel growth peptides
- An antibody against GDNFOS3 was developed

INVENTOR(S)

- [Qing-Rong Liu, PhD \(NIDA\)](#)

DEVELOPMENT STAGE

- Discovery (Lead Identification)

PUBLICATIONS

Airavaara M, et al. Identification of novel GDNF isoforms and cis-antisense GDNFOS gene and their regulation in human middle temporal gyrus of Alzheimer disease. J Biol Chem. 2011 Dec 30;286(52):45093-102.[[PMID 22081608](#)]

PATENT STATUS

- **U.S. Issued:** U.S. 8,999,927

THERAPEUTIC AREA

- Central Nervous System, Mental and Behavioral, Pain